

# PFIZER AIMS TO BECOME INDUSTRY LEADER IN GENE THERAPY WITH ACQUISITION OF BAMBOO THERAPEUTICS, INC.

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Acquisition combines Bamboo's gene therapy portfolio, advanced vector design, and production technologies and capabilities with Pfizer's global scale, research, development and commercialization experience

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Pfizer Inc. (NYSE:PFE) today announced that it has acquired Bamboo Therapeutics, Inc., a privately held biotechnology company based in Chapel Hill, N.C., focused on developing gene therapies for the potential treatment of patients with certain rare diseases related to neuromuscular conditions and those affecting the central nervous system. This acquisition significantly expands Pfizer's expertise in gene therapy by providing Pfizer with a clinical and several pre-clinical assets that complement the company's rare disease portfolio, an advanced recombinant Adeno-Associated Virus (rAAV) vector design and production technology, and a fully functional Phase I/II gene therapy manufacturing facility that Bamboo acquired from the University of North Carolina earlier this year.

Gene therapy is an emerging area of medical research focused on highly specialized, one-time, transformative treatments addressing the root cause of diseases caused by genetic mutation. Gene therapy is a promising investigational technology, especially for patients with rare diseases, many of which are caused by a single genetic mutation. The technology involves introducing genetic material into the body to deliver a corrected copy of a gene to a patient's cells to compensate for a defective one. The genetic material can be delivered to the cells by a variety of means, most frequently using a viral vector such as rAAV. There have been no gene therapy products approved in the U.S. to date.

"The field of gene therapy research has made tremendous strides in recent years, and we are pleased to be able to further enhance our leadership position in this area through this transaction with Bamboo," said Mikael Dolsten, President, Pfizer Worldwide Research & Development. "We believe that gene therapy may hold the promise of bringing true disease modification for patients suffering from devastating diseases, and we hope to see this promise come to fruition – through new and existing in-house capabilities and potential partnership opportunities – in the years to come."

Bamboo's portfolio includes potential best-in-class rAAV-based gene therapies that will complement Pfizer's rare disease and gene therapy portfolios in two priority areas: neuromuscular, with a pre-clinical asset for Duchenne Muscular Dystrophy (DMD); and central nervous system, with pre-clinical assets for Friedreich's

Ataxia and Canavan disease, and a Phase I asset for Giant Axonal Neuropathy.

Bamboo's approximately 11,000-square foot, fully staffed and operational manufacturing facility has experience producing Phase I/II materials using a superior suspension, cell-based production platform that increases scalability, efficiency and purity. This helps enable the DMD program and other projects requiring large amounts of rAAV. The facility, previously known as the University of North Carolina Vector Core facility, has served as a qualified supplier of rAAV vectors for several healthcare companies and academic institutions.

"We believe Bamboo's industry leading capabilities in rAAV vector design and manufacturing complement Pfizer's rare disease strategy and help advance Pfizer's mission to deliver life-changing innovation to patients with the greatest needs," said Gregory LaRosa, Chief Scientific Officer, Rare Disease Research Unit, Pfizer. "Bringing together Pfizer and Bamboo colleagues' deep scientific understanding of both rAAV biology and complex biologic manufacturing will help position us for success in this area. We are pleased to welcome Bamboo colleagues to Pfizer and look forward to working together on transformative gene therapies for patients in areas of high unmet medical need."

Jude Samulski, Chief Scientific Officer and Executive Chairman of Bamboo and a leading expert in the field of rAAV vectors with more than 25 years of experience, will be joining Pfizer. Dr. Samulski, together with the Bamboo team, will play a key role in helping to develop and accelerate Pfizer's capabilities in gene therapy.

"We are pleased to begin working with Pfizer, as this represents a significant step toward bringing Bamboo's portfolio into the clinic and ultimately potential new medicines to patients," Dr. Samulski said.

Pfizer previously acquired approximately 22 percent of Bamboo's fully diluted equity during the first quarter of 2016 for a payment of approximately \$43 million. Under the terms of this transaction, Pfizer acquired all of Bamboo's remaining equity for an upfront payment of \$150 million, and Bamboo's selling shareholders will be eligible for potential milestone payments of up to \$495 million contingent upon the progression of key assets through development, regulatory approval and commercialization. Following the acquisition, Bamboo is now a wholly-owned subsidiary of Pfizer.

Kaye Scholer LLP acted as Pfizer's legal advisor for the transaction and Ice Miller LLP served as Bamboo's legal advisor.

### **Pfizer's Investments in Gene Therapy**

Pfizer is committed to becoming an industry leader in the field of gene therapy, which holds tremendous promise to deliver highly specialized, one-time, transformative therapies to patients in areas of high unmet medical need, particularly in rare, monogenic diseases with loss of function. To support this commitment, the company has been making investments in gene therapy for the past several years and has sought to bring together the foremost expertise in rAAV vector design and development with partnerships, in-house knowledge of disease biology, manufacturing and analytical capabilities.

In 2014, Pfizer established within the company's Rare Disease Research Unit the Genetic Medicines Institute (GMI) in London, UK, which is a dedicated gene therapy research group under the direction of leading gene therapy researcher Michael Linden. Dr. Linden and the GMI are charged with identifying potential gene therapy projects and supporting them through scientific discovery, process development and translational advancement.

In addition to the London-based GMI investment, Pfizer and Philadelphia-based Spark Therapeutics established a collaboration on a clinical program for *SPK-9001*, which is being investigated as a one-time treatment for hemophilia B that incorporates a bio-engineered rAAV vector. Initial data from an ongoing Phase I/II trial for

this treatment has shown promising early results, and *SPK-9001* has received breakthrough therapy designation by the U.S. Food and Drug Administration.

Pfizer has research agreements with several leading academic institutions, including an agreement with King's College London for the development of a series of rAAV gene therapy vectors, and an agreement with the University of Iowa Research Foundation for the development of a potential gene therapy for cystic fibrosis through the University of Iowa laboratories.

Pfizer also entered into a collaboration and license agreement with Emeryville, Calif.-based 4D Molecular Therapeutics (4DMT) to discover and develop targeted next-generation rAAV vectors for cardiac disease. In addition, Pfizer made an equity investment in 4DMT in October 2015.

### **About Pfizer and Rare Diseases**

Rare diseases are among the most serious of all illnesses and impact 350 million patients worldwide, often children. Although there are over 7,000 known rare diseases, only five percent have an approved medication. For rare disease patients and their loved ones, better treatment options cannot come soon enough. At Pfizer, we share their urgency and passionately dedicate our resources, expertise and global reach to bring them the transformative medicines they need. The Pfizer focus on rare diseases builds on more than two decades of experience, a pipeline of more than 20 compounds and a global portfolio of more than 20 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, and pulmonology. Pfizer Rare Disease is inspired by patients, born from science and powered by the passion of the hundreds of colleagues in Pfizer who dedicate their work to helping patients with rare diseases.

### **Pfizer Inc.: Working together for a healthier world®**

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of healthcare products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer healthcare products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. For more information, please visit us at [www.pfizer.com](http://www.pfizer.com). In addition, to learn more, follow us on Twitter at @Pfizer and @Pfizer\_News, LinkedIn, YouTube and like us on Facebook at [Facebook.com/Pfizer](https://www.facebook.com/Pfizer).

### **PFIZER DISCLOSURE NOTICE**

The information contained in this release is as of August 1, 2016. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information related to Pfizer, Bamboo and the acquisition of Bamboo by Pfizer that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Forward-looking statements in this release include, among other things, statements about the potential benefits of the acquisition, Pfizer's and Bamboo's plans, objectives, expectations and intentions, the business of Pfizer and Bamboo, and Pfizer's and Bamboo's rare disease or gene therapy portfolio, technologies and manufacturing capabilities. Risks and uncertainties include, among other

things, risks related to the ability to realize the anticipated benefits of the acquisition, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; the risk that the businesses will not be integrated successfully; unknown liabilities; other business effects, including the effects of industry, market, economic, political or regulatory conditions; future exchange and interest rates; changes in tax and other laws, regulations, rates and policies; the uncertainties inherent in research and development, including the ability to meet anticipated pre-clinical and clinical study commencement and completion dates as well as the possibility of unfavorable study results, including unfavorable new data and additional analyses of existing data; risks associated with initial data; whether and when any applications may be filed with regulatory authorities for any potential rare disease or gene therapy product; whether and when

regulatory authorities may approve any such applications, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted; decisions by regulatory authorities that could affect the availability or commercial potential of any such rare disease or gene therapy product; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2015 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at [www.sec.gov](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).

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